State of California—Health and Human Services Agency
Department of Health Care Services

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DIRECTOR

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Index: Benefits

TO: ALL COUNTY CALIFORNIA CHILDREN SERVICES (CCS) PROGRAM ADMINISTRATORS, MEDICAL CONSULTANTS, AND STATE SYSTEMS OF CARE DIVISION STAFF

SUBJECT: DEFLAZACORT (EMFLAZA™)-REVISED

I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to establish the CCS Program policy regarding the authorization of deflazacort (Emflaza™), as a treatment for Duchenne muscular dystrophy (DMD).

II. BACKGROUND

DMD is a genetic disorder causing progressive muscle deterioration and weakness. This deterioration is caused by the absence or deficient levels of dystrophin protein, which maintains intact muscle cells. DMD primarily affects skeletal, diaphragm, and heart muscle. DMD occurs in about one in 3,600 male infants worldwide with estimated incidence of one in 5,000 male births in the United States. Symptoms appear between ages 3 and 5 and progressively worsen over time. Affected individuals gradually lose their ability to perform daily activities, and are usually wheelchair bound by adolescence, and ventilator dependent by their 20s or 30s.

Deflazacort is a glucocorticoid that first launched in 1985 in Europe, and has been used as an anti-inflammatory, and immunosuppressant in countries outside of the United States since the mid-1990s. On February 9, 2017, it received approval by the Food and Drug Administration (FDA) for the treatment of the signs and symptoms of DMD.

Compared to placebo, corticosteroids including prednisone and deflazacort have been shown to improve strength and pulmonary function[1] in DMD. Compared to prednisone, deflazacort may be associated with less weight gain over the first years of treatment, and greater risk of cataracts.[2,3] The American Academy of Neurology recommends prednisone as the first line steroid treatment for DMD.[3]

III. POLICY

Effective the date of this letter, deflazacort is a CCS Program benefit when the following criteria are met:

A. The client meets the CCS Program residential, financial and medical eligibility criteria.

B. The client has a diagnosis of Duchenne muscular dystrophy, confirmed by genetic testing.

C. The client's care is under the supervision and monitoring of a CCS Program approved Special Care Center (SCC) neurologist or physiatrist.

D. The client is five years of age or older.

E. The request is for the FDA approved indication and does not exceed the FDA approved dosage.

F. The client has been on the recommended dose of prednisone which has been discontinued due to:

   1. Adverse effects

   2. No change in trajectory of disease progression

G. If the client has never been on prednisone, then a trial of prednisone is necessary before re-initiation of deflazacort, unless the prescribing physician provides a compelling justification for exemption from trial of prednisone.

H. For reauthorization of deflazacort, the client's weight or body mass index is stable or less than would be expected recommended dose of prednisone.

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[1] Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016; 87(20):2123-2131.
[2] Bello L, Gordish-Dressman H, Morgenroth LP, et al. Prednisone/prednisolone and deflazacort regimens in the CINRG Duchenne Natural History Study. *Neurology.* 2015; 85(12):1048-1055.
[3] Gloss D, Moxley RT, Ashwal S, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology.* 2016; 86:465-472.

IV. POLICY IMPLEMENTATION

A. Deflazacort (Emflaza™) requires a separate authorization.

B. For initial authorization, requesting providers must submit a CCS Program Service Authorization Request (SAR) to their local county CCS program office or Dependent County Regional Office along with:

1. A copy of the prescription from the CCS Program paneled SCC neurologist or physiatrist.

2. Progress notes from the neurology or rehabilitation SCC documenting laboratory confirmation of DMD, client weight and disease status, and Brooke score or other objective assessment of ambulation.

3. Documentation of prior prednisone use and reason for discontinuation, or documentation explaining why prednisone is not an option.

4. County and regional office staff shall pend a service authorization request when the required documentation has been submitted.

5. All requests shall be reviewed by a CCS Program State Medical Consultant in consultation with a local county CCS program Medical Director or designee before authorization of deflazacort.

6. Initial authorization shall be for six months.

C. For reauthorization, requesting providers must submit a CCS Program Service Authorization Request (SAR) to their local county CCS program office or Dependent County Regional Office along with:

1. Body mass index or weight before and after deflazacort use.

2. Documentation of client’s ambulatory status such as Brooke Score.

3. For clients previously on prednisone, documentation must substantiate that prednisone related adverse effects have resolved with deflazacort use.

4. Documentation of ophthalmology follow-up at least annually for surveillance and medical follow-up for potential cataracts.

5. Report by registered dietician.

6. Reauthorization shall be for up to 12 months.

7. The State CCS Program Medical Director or designee will review exceptions on a case-by-case basis.

If you have any questions regarding this N.L., please contact Jill Abramson, M.D. M.P.H, Chief, Medical Policy & Consultation Section, at (916) 327-2108 or via e-mail at Jill.Abramson@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY

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